EXHIBIT A

Record 1 of 7

Recruiting

An Open-Label ProSpective MultiCENTer Study to Evaluate Safety and Tolerability of Dry Powder Inhaled Treprostinil in PH (**ASCENT**)

ClinicalTrials.gov ID NCT06129240

Sponsor Liquidia Technologies, Inc.

Information provided by Liquidia Technologies, Inc. (Responsible Party)

Last Update Posted 2024-10-03

Researcher View Tab

Trial Contacts

Contacts *
Not provided

Study Record Dates

First Submitted
2023-11-06

First Posted

2023-11-13

Last Update Posted

2024-10-03

Last Verified *

2024-10

Outcome Measures

Change History

See all versions of this study

Primary (Current) *

(Submitted: 2023-11-10)

- Number of participants with treatment-emergent drug/device-related adverse events and Serious Adverse Events (SAEs) [Time Frame: Baseline until the end of the study, approximately 2.5 years, June 2026]
  - Treatment-emergent adverse events and serious adverse events will be grouped by MedDRA System Organ Class, dose level at onset, time on drug at onset, and relationship to dose titration

Primary (Original) *

Same as current

Secondary (Current) [*]

Not provided

Secondary (Original) [*]

Not provided

Other Pre-specified (Current)

Not provided

Other Pre-specified (Original)

Not provided

Trial Description

Brief Title *

An Open-Label ProSpective MultiCENTer Study to Evaluate Safety and Tolerability of Dry Powder Inhaled Treprostinil in PH

Official Title *§

An Open-Label ProSpective MultiCENTer Study to Evaluate Safety and Tolerability of Dry Powder Inhaled Treprostinil in **Pulmonary Hypertension**

Brief Summary *

Study LTI-401 is an open-label, multicenter study which will evaluate the safety and tolerability of LIQ861 in subjects who have WHO Group 1 & 3 PH.

Detailed Description

Study LTI-401 is an open-label, multicenter study which will evaluate the safety and tolerability of LIQ861 in subjects who have WHO Group 1 & 3 PH.

Cohort A will include approximately 60 subjects who have WHO Group 3 Pulmonary Hypertension associated with interstitial lung disease (PH-ILD)

Additional cohorts from either Group 1 or Group 3 may be defined in future protocol amendments.

Scheduled study visits to the clinic will occur at Screening, Baseline, Week 8, Week 16, Week 24, and Week 52. During this time, dose titration may be ordered at the Investigator's discretion and in accordance with the guidance provided.

The primary objective of this study is to evaluate the safety and tolerability of LIQ861 in subjects with WHO Group 1 & 3 Pulmonary Hypertension (PH).

The exploratory objectives of the study are to assess the effects of LIQ861 on exercise capacity, functional class, relevant biomarkers, and imaging assessments.

Study Type *

Observational [Patient Registry]

Study Design *

Observational Model
Cohort

Time Perspective
Prospective

Target Follow-up Duration *

52 Weeks

Biospecimen

Not provided

Sampling Method *

Non-Probability Sample

Study Population *

Subject with PH-ILD

Condition *

- **Pulmonary Hypertension**
- Interstitial **Lung** Disease

Intervention

- Combination Product: LIQ861
  - trepostinil inhalation powder

Groups/Cohorts

- Cohort A
  - PH-ILD
  - Interventions:
    - Combination Product: LIQ861

Publications

(Includes general and study results' publications, and Pubmed publications referencing this study by ClinicalTrials.gov Identifier (NCT Number))

Not provided

Recruitment Information

Recruitment Status *

Recruiting

Enrollment (Estimated) *

(Submitted: 2023-11-10)

60

Original Enrollment (Estimated) *

Same as current

Study Start Date (Actual) *§

2023-12-28

Primary Completion Date (Estimated) *

2026-07-30 (Final data collection date for primary outcome measure)

Study Completion Date (Estimated) *§

2026-10-31

Eligibility Criteria *

Inclusion Criteria:

1. An Institutional Review Board (IRB) approved informed consent is signed and dated by the subject prior to any study related activities.
2. Male or Females between 18 years to 75 years of age at Screening. Subjects between 76 and 80 years of age at Screening can be considered for eligibility but will require approval of the Sponsor's MM or designee.
3. If the subject is a female of childbearing potential, then the subject must have a negative pregnancy test result at the Baseline Visit and agrees to practice adequate birth control throughout the duration of the study. If the subject is postmenopausal or has documented surgical sterilization, a pregnancy test and birth control are not necessary. It is the Investigator's responsibility for determining whether the subject has adequate birth control for study participation.

4. Has a confirmed diagnosis of WHO Group 3 PH-ILD based on CT chest imaging performed within 12 months prior to baseline visit, which demonstrates evidence of diffuse parenchymal lung disease and FEV1/FVC (absolute values) >70%. Subjects are required to have evidence of pulmonary hypertension (PH) as demonstrated from right heart catheterization (RHC) within 6 months of the baseline visit with the following documented parameters depending on their lung disease category a) or b).

   a. Subjects may have any form of ILD or combined pulmonary fibrosis and emphysema (CPFE).

   i) Pulmonary vascular resistance (PVR) > 3 Wood Units (WU) and ii) Pulmonary capillary wedge pressure (PCWP) of < 15 mmHg and iii) A mean pulmonary arterial pressure (mPAP) of > 30 mmHg

   OR b. A exploratory subset of subjects with ILD: i) Pulmonary vascular resistance (PVR) > 3 Wood Units (WU) and ii) Pulmonary capillary wedge pressure (PCWP) of < 15 mmHg and iii) A mean pulmonary arterial pressure (mPAP) of > 21 mmHg

5. Must be able to walk a distance of >125 meters on two six-minute walk tests (6MWTs) during the screening and baseline assessments. The variability of the distances between the two qualifying 6MWTs must be within 15% of one another, calculated from the furthest walk.
6. Subjects on a chronic medication for underlying lung disease (i.e. pifenidone, nintedanib, etc) must be on a stable dose and regimen for > 30 days prior to Baseline and planned to continue for the duration of the study at the same dose.
7. Demonstrates the ability to use the RS00 Model 8 inhaler.

Exclusion Criteria:

A Subject is not eligible for inclusion in the study if any of the following criteria apply:

1. Pulmonary hypertension (PH) in the Updated WHO Classification Groups 1, 2, 4, or 5.
2. Intolerance or significant lack of efficacy to a prostacyclin or prostacyclin analogue that resulted in discontinuation or inability to effectively titrate that therapy.
3. Received any FDA approved PAH or PH-ILD prostacyclin therapy including: epoprostenol, treprostinil, iloprost, beraprost, or IP receptor agonist (selexipag), except for acute vasoreactivity testing within 60 days of Screening.

4. Received any FDA PAH approved oral therapy including: endothelin receptor antagonist (ERA), or soluble guanylate cyclase (sGC) stimulator within 60 days of Baseline.

   - phosphodiesterase type 5 inhibitors (PDE5-I) are permitted if on a stable dose for at least 60 days prior to the right heart catheterization and planned to continue for the duration of the study at the same dose.
   - PDE5-I are permitted for erectile dysfunction as needed but should be withheld at least 48 hours prior to a scheduled visit.

5. New type of chronic therapy (including but not limited to oxygen, a different class of vasodilator, diuretic, digoxin, and digitalis) for pulmonary hypertension added within 30 days of Screening and prior to Baseline.
6. Uncontrolled systemic hypertension as evidenced by persistent, systolic blood pressure greater than 160 mmHg or diastolic blood pressure greater than 100 mmHg.
7. History of hemodynamically significant left-sided heart disease including, but not limited to: aortic or mitral valvular disease more than mild, pericardial constriction, restrictive or congestive cardiomyopathy with estimated left ventricular ejection fraction less than 40%, or symptomatic coronary artery disease (CAD).
8. Prior atrial septostomy.
9. Receiving > 8 L/min of oxygen supplementation by any mode of delivery at rest at Screening and prior to Baseline.
10. Serious or life-threatening disease other than conditions associated with PH or ILD.
11. Therapy with any excluded medications listed in the Investigator's Brochure.
12. Hypersensitivity or allergy to any of the ingredients of LIQ861 or other clinically relevant allergies (clinical relevance per Investigator judgment).
13. Exacerbation of underlying lung disease or active pulmonary or upper respiratory infections within 30 days of Screening and prior to Baseline.
14. Current RT-PCR confirmed severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) infection or recent history of lab confirmed SARS-CoV-2 infection in previous 30 days of Screening and prior to Baseline.
15. ILD developed from a SARS-CoV-2 infection (ILD should have preceded infection).
16. Initiation of pulmonary rehabilitation within 12 weeks prior to Screening (participants who are stable in the maintenance phase of a program and who will continue for the first 16 weeks of the study are eligible).
17. In the opinion of the Investigator, the subject has any condition that would interfere with the interpretation of study assessments or has any disease or condition (i.e., peripheral vascular disease, musculoskeletal disorder, morbid obesity) that would likely be the primary limit to ambulation (as opposed to PH).
18. Acute pulmonary embolism within 90 days of Screening or prior to Baseline.
19. Stroke or transient ischemic attack (TIA) within six months of Screening or prior to Baseline.
20. Evidence of an active uncontrolled sepsis or systemic infection during Screening.
21. Pregnant, plans to become pregnant, or lactating.
22. Participated in an investigational drug or device study within the 30 days prior to Screening.
23. In the opinion of the Investigator, significant use of any inhaled tobacco/marijuana products or significant recent history of drug abuse at the time of informed consent.
24. Subject has severe hepatic impairment as evidenced by any history of ascites AND encephalopathy.
25. Renal impairment (eGFR < 40). (Appendix 5).
26. Severe concomitant illness limiting life expectancy (≤ 6 months) or listed "active" for lung transplantation.
27. Currently on hospice status or planned hospice status in the next 6 months.

28. Known anaphylactoid reaction or hypersensitivity allergy to intravenous iodinated contrast media.

Sex/Gender *

Sexes Eligible for the Study:
All

Ages *

18 Years to 80 Years (Adult, Older Adult)

Accepts Healthy Volunteers

No

Location Countries

United States

Removed Location Countries

Administrative Information

NCT Number

NCT06129240

Other Study ID Numbers [*]

LTI-401

Has Data Monitoring Committee

Not provided

U.S. FDA-regulated Product

Studies a U.S. FDA-regulated Drug Product
Yes

Studies a U.S. FDA-regulated Device Product
No

IPD Sharing Statement

Not provided

Current Responsible Party *

Liquidia Technologies, Inc.

Original Responsible Party *

Same as current

Current Study Sponsor *

Liquidia Technologies, Inc.

Original Study Sponsor *

Same as current

Collaborators

Not provided

Investigators

Not provided

PRS Account

Liquidia Technologies, Inc.

Symbol Legend

*	Required
*§	Required if Study Start Date is on or after January 18, 2017
[*]	Conditionally required
No Symbol	Unmarked fields are optional. If no information is provided, fields will be labeled as "not provided"